Maryland
DEPARTMENT OF HEALTH

Exondys 51 (eteplirsen)

Exondys 51 is an antisense oligonucleotide indicated for the treatment of Duchenne Muscular Dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping.

I. Criteria for Initial Approval

Exondys 51 will be considered for coverage when **all** of the criteria below are met, confirmed with supporting medical documentation.

- Treatment with Exondys 51 should be initiated, in male patients, before the age of 14 years.
- Prescribed by, or in consultation with a neurologist who specializes in treatment of DMD.
- Patients must have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping.
- Patient has been on a stable dose of corticosteroid, unless contraindicated or intolerance, for at least 6 months.
- Documentation of the following pretreatment status:
    - Patient retains meaningful voluntary motor function.
    - Ambulatory status:
        - Ambulatory, with or without assistance devices, with 6 minute walk test (6MWT) of 180 m or greater (6MWT must be done within prior month).
    - Respiratory status:
        - Not ventilator dependent.
- Patient receiving ongoing physical therapy.
- Provider attestation of baseline and subsequent evaluation and monitoring as appropriate, (e.g., hypersensitivity reactions and renal function).
- Patient is not receiving other RNA antisense therapy or gene therapy for DMD.

II. Criteria for Continuation of Therapy

All of the criteria for initial therapy (in **Section I**.) must be met; **AND** the provider must attest to a positive clinical response.

A positive clinical response is defined when the patient has responded to therapy compared to the pretreatment baseline in one or more of the following categories;

- Stability, improvement, or slowed rate of decline in ambulatory function as demonstrated by the Six Minute Walk Test - 6MWT.
- Stability, improvement, or slowed rate of decline in respiratory function.
- Improvement in quality of life.

III. Dosing/Administration

Exondys 51 must be administered according to the current FDA labeling guidelines for dosage and timing. The recommended dosing is as follows:

- 30 milligrams per kilogram of body weight, one time, weekly.

IV. Length of Authorization for Therapy

Exondys 51 will be authorized for 6 months when criteria for initial approval are met. Continuing therapy for Exondys 51 will be authorized for 12 months.

V. Billing Code/Information

J1428 - Injection, eteplirsen, 10 mg; 1 billable unit = 10 mg.

Prior authorization of benefits is not the practice of medicine nor the substitute for the independent medical judgment of a treating medical provider. The materials provided are a component used to assist in making coverage decisions and administering benefits. Prior authorization does not constitute a contract or guarantee regarding member eligibility or payment. Prior authorization criteria are established based on a collaborative effort using input from the current medical literature and based on evidence available at the time.

Approved by MDH Clinical Criteria Committee: 2/23/2021
Last Reviewed Date: 2/23/2021